NFlection Therapeutics Announces Positive Results from Phase 2b Study of NFX-179 Topical Gel in the Treatment of Cutaneous Neurofibromas in Neurofibromatosis Type 1

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NFlection Therapeutics →
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- *Once-daily topical treatment of target cutaneous neurofibromas (cNFs) with NFX-179 Gel 1.5% achieved statistically significant reduction in size*
- *First randomized, double-blind, vehicle-controlled Phase 2b clinical trial to report positive results in the treatment of cutaneous neurofibromas*
- *Multiple secondary endpoints met; significant reductions in the volume and height of cNFs correlates with patient-reported meaningful improvement in their cNFs*
- *Efficacy and tolerability results support advancing NFX-179 Gel program to Phase 3 development in 2024*

BOSTON, Nov. 13, 2023 /PRNewswire/ -- NFlection Therapeutics Inc., a company developing topical MEK inhibitors for RAS-mediated skin conditions, today announced positive topline results from a randomized, double-blind, vehicle-controlled Phase 2b clinical trial (NCT05005845) evaluating NFX-179 Gel as a treatment for cutaneous neurofibromas (cNFs) in people with neurofibromatosis type 1 (NF1), a rare genetic condition. Cutaneous neurofibromas appear in over 95% of people with NF1 and are physically disfiguring, painful, cause extensive psychological harm, and negatively impact quality of life. There are currently no FDA-approved therapeutics for the treatment of cNFs.

The Phase 2b study was a 6-month double-blind, randomized, vehicle-controlled study of 199 subjects to determine the safety and effectiveness of NFX-179 Gel 0.5% and 1.5% compared with vehicle in patients with cNFs. The study was conducted at 24 investigational centers in the U.S.

NFX-179 Gel was applied topically to target cNF tumors for six months, enabling delivery of high concentrations of NFX-179 to the tumors. The highest concentration of NFX-179 tested, 1.5%, achieved the study's primary efficacy endpoint, with a statistically significant improvement over vehicle in the shrinkage of cNFs in people with NF1 that was highly correlated with patient-reported meaningful improvements in their cNFs. Consistent with its design as a soft, or metabolically labile, MEK inhibitor, NFX-179 Gel was generally well tolerated, and plasma drug concentrations were orders of magnitude lower than those typically observed for oral MEK inhibitors.

"Cutaneous neurofibromas, which present in over 95 percent of people with NF1, are associated with significant psychosocial impact and are often the most burdensome symptom of the condition," said study investigator Miriam Bornhorst, MD, Assistant Professor of Pediatrics, and the Clinical Director of the Gilbert Family Neurofibromatosis Institute at Children's National Hospital. "The data shows that NFX-179 Gel significantly reduced the size of cNFs across multiple efficacy endpoints and was generally well tolerated. We are excited to see the potential for NFX-179 Gel as the first topical therapy for the treatment of cNFs in people with NF1."

The primary efficacy endpoint of the study was a subject-level responder analysis, with a response defined as at least 50% reduction in cNF volume above the surrounding non-tumor skin for five or more of the ten treated tumors, after 6 months of once-daily application of NFX-179 Gel.

The study met the primary efficacy endpoint with dose-dependent responder rates of 44.2% with NFX-179 Gel 1.5%, 34.8% with NFX-179 Gel 0.5%, and 24.1% with vehicle. The high dose had a statistically significant higher responder rate than vehicle ($p = 0.03$).

"We are pleased to report success from our Phase 2b NF1 patient trial that supports the first-in-class potential of NFX-179," said William Hodder, Chief Executive Officer of NFlection. "Based on the favorable efficacy and tolerability profile NFX-179 Gel demonstrated in this study, we plan to meet with U.S. and European regulators and move the program to Phase 3

development in 2024. We would like to thank the patients, our investigators and collaborators, including The Children's Tumor Foundation, who made this study possible. We look forward to continuing the development of this important therapy for the NF1 community."

"In addition to developing efficient preclinical and clinical neurofibromatosis (NF) test platforms, CTF is dedicated to investing in new medicines for people with NF. Our support for NFlection demonstrates that commitment," said Annette Bakker, PhD, President of The Children's Tumor Foundation. "People with NF1 with cutaneous neurofibromas have been overlooked for a long time and are desperate for safe and easy-to-apply treatment options."

About NFX-179 Topical Gel

NFX-179 is an investigational mitogen-activated protein kinase kinase (MEK) inhibitor. NFX-179 is a "soft" (metabolically labile) drug, which, when formulated as NFX-179 Gel for topical application, is designed to concentrate at the dermal site of action but degrade in systemic circulation, thereby significantly reducing side effects routinely seen with systemically available MEK inhibitors. NFlection is developing NFX-179 Gel for the treatment of cutaneous neurofibromas in people with neurofibromatosis type 1 and has received Orphan Designation in the United States and European Union for this indication.

About NFlection Therapeutics Inc.

NFlection is a clinical-stage biopharmaceutical company focused on the development of novel therapies to address the needs of patients with neurofibromatosis type 1, immunosuppressant-mediated squamous cell carcinoma, and congenital birthmarks such as keratinocytic epidermal nevi and nevi sebacei. To address these RAS-mediated disorders driven by the aberrant activation of the Ras/Raf/MEK/ERK pathway, we are developing first-in-class MEK (mitogen-activated protein kinase kinase) inhibitors for topical treatment of these conditions. To learn more about the company, please visit www.nflection.com.

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